CIRM HIV/AIDS disease team technology makes news

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Richmond-based Sangamo BioSciences has been making a lot of news lately with their gene editing technology. Theirs is the technique being used in CIRM'S HIV Disease Team Award to John Zaia at The City of Hope (summarized in this San Francisco Business Journal story).

Sangamo's so-called zinc finger technology can recognize a specific location in the DNA, snip it out, and replace it with a different sequence. In the case of HIV, the molecular zinc scissors are being used to create a mutation in a small region of DNA in blood-forming stem cells.

Those cells altered in the lab lack a working copy of the protein CCR5, which the HIV virus uses to enter and destroy immune cells. The team then plans to transplant those altered stem cells into a person, where they create a new immune system that is resistant to HIV infection. Early results from this work in animals look promising and the team is hoping to be able to enter human clinical trials with the technique in the next few years.

This is one of two CIRM disease teams attempting to generate a stem cell-based therapy for HIV/AIDS. The other award, to Irvin Chen at UCLA, is using a different type of molecule to mutate the CCR5.

Ron Leuty of the San Francisco Business Journal had a story yesterday about Sangamo's prospects, which include a trial to treat pain associated with diabetes, called diabetic neuropathy. The technique is also being used in research to treat the blood-clotting disease hemophilia B and to create disease-in-a-dish models of heart disease. Reuty wrote about the heart disease work, being carried out by Sangamo and researchers at the Scripps Translational Science Institute:

“ Using induced pluripotent stem cells - adult stem cells manipulated to give them embryonic-like qualities - researchers will recreate cells that line the arteries. ...

“ "Genome editing allows us to do an experiment no one has ever tried - that is, if you change someone's genetics, can you make their cells revert away from acquiring a disease?" Samuel Levy, director of genomic sciences at the Scripps Translational Science Institute, said in a press release.

This video describes how the City of Hope team hopes to use the zinc finger technology in their proposed therapy for HIV.

You can also watch talks by City of Hope research John Zaia, CIRM board member and HIV patient advocate Jeff Sheehy, and HIV advocate Loren Leeds when they spoke to the CIRM governing board about the work.

-A.A.

Tags: sheehy, Disease Team, HIV/AIDS, Zaia, leeds, sangamo, city of hope

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